

## IN VIVO LENTIVIRAL GENE THERAPY FOR THE TREATMENT OF PRIMARY HYPEROXALURIA TYPE-1

The present Technology developed by researchers from CIBER, CIEMAT and FIIS-FJD provides a method for the treatment of PH1

### The Need

Primary Hyperoxaluria Type 1 (PH1) is a disease of the glyoxylate metabolism with autosomal recessive inheritance caused by a deficient activity in the liver enzyme alanine glyoxylate aminotransferase (AGT) due to mutations in the AGXT gene. This enzymatic deficiency causes an excessive hepatic production of oxalate and its consequent accumulation in the kidney, which leads to chronic kidney damage.

### The Solution

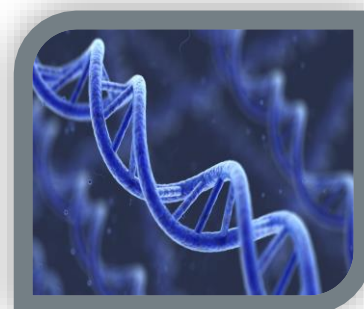
Currently, gene therapy for inherited metabolic liver diseases is mostly based on in vivo approaches. In this context, LVs represent an opportunity to address these challenges. LVs are already on the market in gene therapy products with autologous transduced hematopoietic stem/progenitor cells or T cells.

### Innovative Aspects

Since around 30% of PH1 patients have an infantile onset it becomes crucial to develop a gene therapy strategy with the potential to persist life-long, following a single administration to pediatric patients.

The main hypothesis of this proposal is that the systemic administration of a lentivirus (LV) that expresses an enhanced version of AGXT gene with a liver-specific expression pattern in patients with PH1 can correct a sufficient amount of hepatocytes required for limiting the oxalate overproduction before the onset of the renal damage.

In this way, this project will contribute to the development of a potential definitive cure for PH1 and to other inherited liver metabolic diseases.



### Stage of Development:

Preliminary experiments have been conducted to evaluate the feasibility of an in vivo gene therapy for the treatment of PH1, which encourage the advance of this project.

### Intellectual Property

- ❖ Priority European patent application filed
- ❖ Suitable for international extension (PCT application)

### Aims

Looking for a partner interested in a license and/or collaboration agreement to develop and exploit this asset

### Contact details